

Vision for the NIH and the Role of the Council of Councils

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NIH Director

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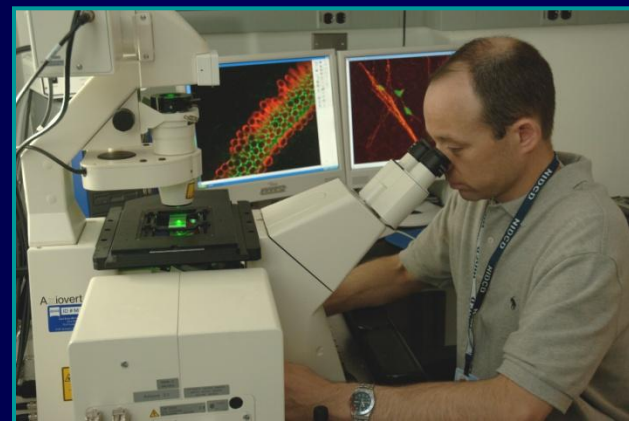
My first 97 days as NIH Director.....



NIH: Steward of Medical and Behavioral Research for the Nation



“Science in pursuit of **fundamental knowledge** about the nature and behavior of living systems... and the **application of that knowledge** to extend healthy life and reduce the burdens of illness and disability.”

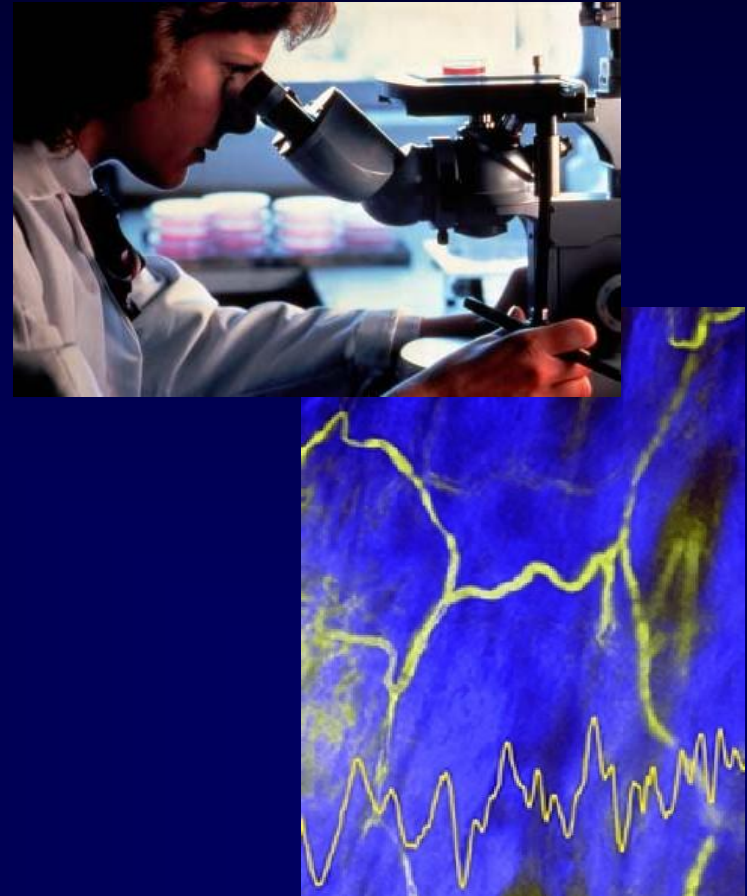


Investigator-Initiated Research:

The Foundation of Biomedical Advances

“... the hope of major advances lies in sustaining broad and free-ranging inquiry into all aspects of the phenomena of life, limited only by the criteria of excellence, the scientific importance, and the seriousness and competence of the investigator.”

**– James A. Shannon, M.D.
8th Director of NIH**



Opportunities in Biomedical Research

- Opportunity #1:

Applying unprecedented opportunities in genomics and other high throughput technologies to understand fundamental biology, and to uncover the causes of specific diseases



Opportunities in Biomedical Research

- **NextGen DNA sequencing and beyond**
- **Nanotechnology**
- **Small molecule screening**
- **New imaging modalities**
- **Emphasis on comprehensive approaches**
- **Computational biology is critical**
- **Examples that are ripe for expanded effort**
 - Cancer
 - Autism
 - Microbiome
 - Many more....

Opportunities in Biomedical Research

- Opportunity #2:

Translating basic science discoveries into new and better treatments

- Moving from...



Opportunities in Biomedical Research

- To...



Opportunities in Biomedical Research

- **Stage set for NIH to play an expanded role in translation**
 - New discoveries about the fundamental basis of disease
 - Resources empowering academic investigators to develop lead compounds and “de-risk” projects
 - Opportunity for public-private partnerships
- **Small molecules**
 - Roadmap provides HTS capabilities that now exceed that of most pharmaceutical companies
 - Many ICs are ramping up investments
 - Therapeutics for Rare and Neglected Diseases (TRND) program now explicitly tackles the Valley of Death
 - Common diseases are increasingly seen as a collection of rare diseases, so they fit this model too
- **Stem cells (including hESCs and iPSCs)**
 - Explosion of new information likely with new NIH policy
 - Therapeutic uses uncertain but urgent to pursue

Opportunities in Biomedical Research

- Opportunity #3:
Putting science to
work for the benefit of
health care reform



Opportunities in Biomedical Research

- **Comparative effectiveness research**
- **Prevention and personalized medicine**
 - Behavioral research
- **Health disparities research**
- **Pharmacogenomics**
- **Large scale prospective studies**
 - National Children's Study
 - Study of adult onset diseases?
- **Health IT**
- **Health economics research**

Opportunities in Biomedical Research

- Opportunity #4:
Encouraging a
greater focus on
global health



Opportunities in Biomedical Research

- **Emphasis on global health fits with U.S. emphasis on “soft power”**
- **Scientific advances make an attack on infectious diseases more feasible than ever**
 - RNAi
 - Small molecule screening
 - Genomics
 - Vaccine development
- **Push beyond AIDS, malaria, TB to long list of neglected tropical diseases (NTDs) affecting hundreds of millions of people worldwide**
- **Include chronic non-communicable diseases and injuries, responsible for more 50% of deaths in developing world**

Opportunities in Biomedical Research



NIH Roadmap for Medical Research

- Opportunity #5:
Reinvigorating and
empowering the
biomedical research
community



Opportunities in Biomedical Research

- **Strive to avoid the post-ARRA cliff**
- **Reinvigorating the Common Fund**
- **Emphasis on innovation**
 - Pioneer awards
 - New Innovator awards
 - Transformative RO1s
- **Assessing results of current innovations in peer review**
- **Training programs**
 - Seek to reduce age at first grant award
 - Underrepresented minority programs
 - Work force analysis – what's the right answer?

Advisory Committees

- **Four key external committees advise the NIH director:**
 - **Advisory Committee to the NIH Director**
(NIH Director co-Chairs)
 - **Council of Public Representatives**
(NIH Director co-Chairs)
 - **Scientific Management Review Board**
(NIH Director is ex officio)
 - **Council of Councils:** *Advises the NIH Director*

**“A committee is a cul-de-sac
down which ideas are lured and
then quietly strangled.”**

**Sir Barnett Cocks
(1907 -1989)**

**“It is better to give than to
receive – especially advice.”**

**Mark Twain
American Author and Lecturer
(1835 – 1910)**

Advisory Committee to the NIH Director

- **Focus:**
 - Program development
 - Resource allocation
 - NIH administrative regulation and policy
 - Other aspects of NIH policy
- **Specific Activity:**
 - Advises on grant applications and cooperative agreements for research and training



Council of Public Representatives

- **Focus:**
 - Public participation in NIH activities
 - Outreach and public communication efforts
 - Other matters of public interest
- **Specific Function:**
 - Provides a formal mechanism for public input into the research decision-making and priority-setting process



Scientific Management Review Board

- **Focus:**
 - Ensuring the optimal organizational structure and function for the fulfillment of NIH mission
- Recommendations are provided to NIH, HHS, and Congress



Council of Councils

- **Focus:**
 - Policies and activities of the Division of Program Coordination, Planning, and Strategic Initiatives
- **Specific Activities:**
 - Advise on research responsive to emerging scientific opportunities, public health challenges, and knowledge gaps
 - Advise the IC Directors during review process for trans-NIH initiatives

Mandate for the Council of Councils

- The Council was established by the NIH Reform Act of 2006.
- The Council's mandate is to advise me on DPCPSI policies and activities.

One Hundred Ninth Congress
of the
United States of America

AT THE SECOND SESSION

*Begun and held at the City of Washington on Tuesday,
the third day of January, two thousand and six*

An Act

To amend title IV of the Public Health Service Act to revise and extend the authorities of the National Institutes of Health, and for other purposes.

*Be it enacted by the Senate and House of Representatives of
the United States of America in Congress assembled,*

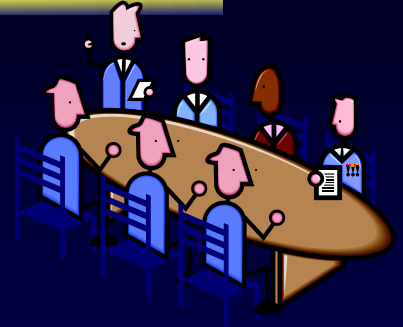
SECTION 1. SHORT TITLE.

This Act may be cited as the "National Institutes of Health Reform Act of 2006".

TITLE I—NIH REFORM

Role of the Council of Councils

- Your job is to bring your knowledge of the ICs' missions and operations to the CoC -- not to serve as the official representatives from the IC Councils.
- May or may not report back to your IC Council on the efforts of this body.
- We need you to explore and provide advice beyond the research agenda of any one IC.

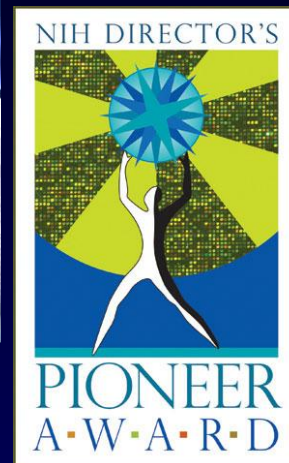


Past Advice from the Council

- The Council has been asked to advise on:
 - The activities of the former OPASI
 - The activities of DPCPSI, such as RCDC system and the TR01 Program
 - Provide second-level review of the Transformative R01 grant applications
 - Early concept clearance of programs proposed for Common Fund support

The Common Fund in FY 2010: 13 programs covering the spectrum of the NIH mission

- **Clinical Research**
 - CTSAs
- **High Risk/High Reward**
 - Pioneers, New Innovators, TR01s
- **Resources to foster discovery and translation**
 - Molecular Libraries, Nanomedicine, ...
- **Interdisciplinary Research**
- **New**
 - Microbiome, Genotype/Tissue Expression Resources (GTEx), ...



Common Fund Forward: Demands and Needs

- **Ensure continuing support for existing Roadmap projects.**
- **Focus on future uses of the Common Fund.**
- **Common Fund commitment base is high – setting tight limits on new projects that can be initiated over next 2 years.**
- **Identify new ways for the Common Fund to be more facile.**
- **Select compelling projects for support.**

Proposed New Efforts for Common Fund Support

- TRND/RAID expansion
- Regulatory/Translational Science
- Neglected Tropical Diseases/Global Health
- STEM Education
- Expansion of the Genotype-Tissue Expression Project
- Connectivity Map (LINCS)

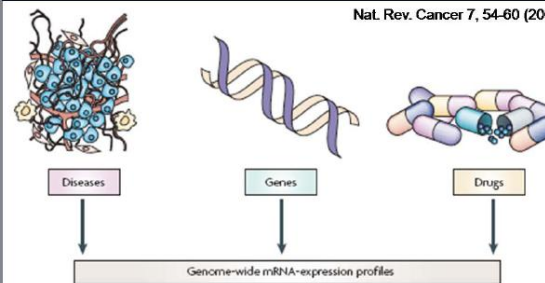


The Connectivity Map: Using Gene-Expression Signatures to Connect Small Molecules, Genes, and Disease

Justin Lamb,^{1,*} Emily D. Crawford,^{1,†} David Peck,¹ Joshua W. Modell,¹ Irene C. Blat,¹ Matthew J. Wrobel,¹ Jim Lerner,¹ Jean-Philippe Brunet,¹ Aravind Subramanian,¹ Kenneth N. Ross,¹ Michael Reich,¹ Haley Hieronymus,^{1,2} Guo Wei,^{1,2} Scott A. Armstrong,^{2,3} Stephen J. Haggarty,^{1,4} Paul A. Clemons,¹ Ru Wei,¹ Steven A. Carr,¹ Eric S. Lander,^{1,5,6} Todd R. Golub^{1,2,3,5,7*}

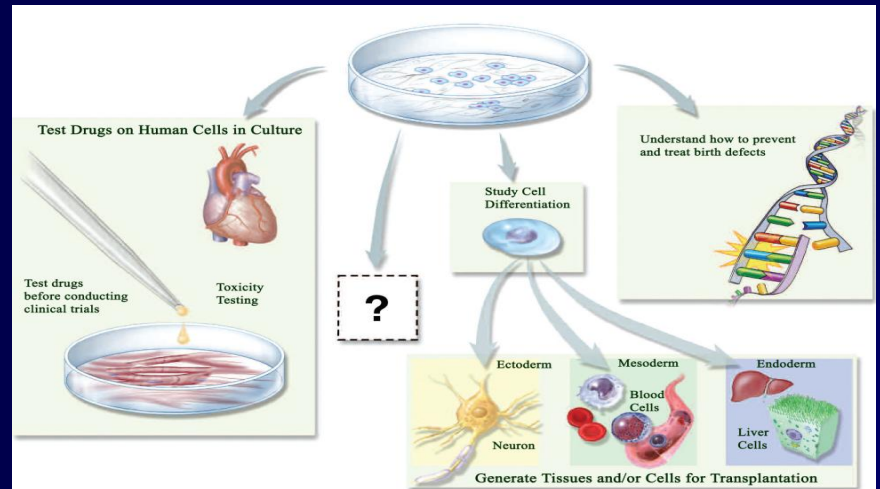
Science 313, 1929-1935 (2006)

Nat. Rev. Cancer 7, 54-60 (2007)



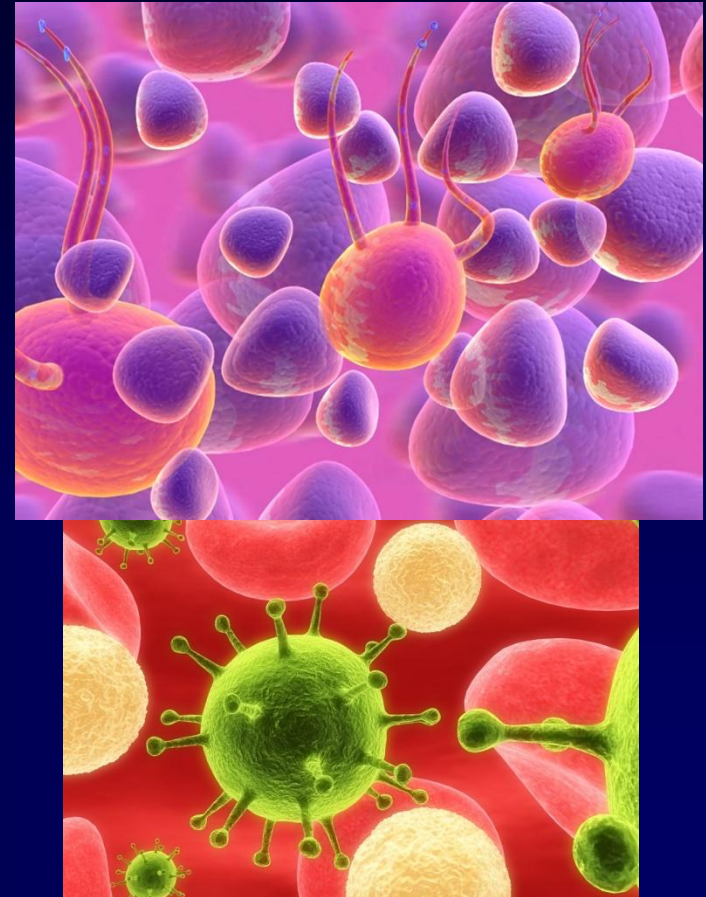
Proposed New Efforts for Common Fund Support (continued)

- Biobanks Workshop
- Health Economics Research
- Protein Affinity Reagents
- Mouse Phenotyping
- HMO Collaboratory
- Translational Stem Cell Research
- Science of Behavior Change



Concept Proposals Previously Reviewed by the Council

- We may pursue aspects of these proposals in the context of proposed new efforts
- LINCS and MAPGen seek to understand how gene networks operate in diverse cell types and how a single faulty network can underlie apparently distinct diseases
- Current consideration for a Connectivity Map aligns well with these goals – it aims to perturb networks and measure the cellular impact in a number of cell types.



Concept Proposals Previously Reviewed by the Council

- **An initiative similar to the Clinical Impact Awards**
- **Therapeutics for Rare and Neglected Diseases (TRND)**
– is a new Trans-NIH program to facilitate drug discovery and development.



Seeking Far-Reaching Common Fund Ideas

- Should we convene brainstorming meetings with stakeholders, i.e., create “Grand Challenges” to design far-reaching efforts with the potential to transform health and medicine?
- Focus would need to be on projects for FY12 and beyond



Role of the Council of Councils

- **We will continue to seek your counsel on these efforts**
- **We welcome your ideas for research initiatives to gain new knowledge with the goal of improving health – here and around the world**
- **We are about to announce a search for a permanent DPCPSI Director**

Not failure, but low aim, is a crime.

— *James Russell Lowell*